SCIENTIFIC ABSTRACT

Adult patients with X-linked chronic granulomatous disease (CGD) will undergo peripheral blood progenitor cell (PBPC) harvesting following granulocyte-colony stimulating factor (G-CSF) mobilization. $15x10^8$ mononuclear cells (MNC)/kg will be collected by apheresis. CD34+ cells will be isolated utilizing the Isolex™300i system developed by Baxter Healthcare Corporation. CD34+ cells will be transduced with the human $gp91^{phox}$ cDNA in the retroviral construct, MSCV-h91Neo. Following transduction, patients will be infused with one-half of the freshly transduced CD34+ cells and the remaining half will be cryopreserved with reinfusion six months after transduction. After each infusion, patients will receive G-CSF on Days 18-20 post-infusion to ascertain whether the absolute number of transduced neutrophils and monocytes are increased. Various assays for the presence and expression of the $gp91^{phox}$ cDNA will be performed on peripheral blood and bone marrow along with safety testing. Safety testing is discussed and described in detail in the body of the protocol.